CIRM-funded Clinical Trial Aimed at Blocking HIV/AIDS in People Gets the Go Ahead

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San Francisco, CA – An innovative therapy using a patient's own stem cells, modified to resist infection with the AIDS virus, has been given approval by the Food and Drug Administration (FDA) to begin a clinical trial in people. CIRM, California's stem cell agency, is funding that trial.

The therapy focuses on a protein called CCR5, which is found on cells in the immune system. The AIDS virus hijacks this protein, using it to penetrate and infect our cells.

Researchers at City of Hope near Los Angeles - working with Keck Medicine of the University of Southern California (USC) and Sangamo BioSciences (a clinical stage biopharmaceutical company involved in genome editing and gene therapy) - plan to take blood stem cells from HIV infected individuals, then treat them with zinc finger nucleases (ZFNs), a kind of molecular scissors, to disrupt the CCR5 gene in those cells. The hope is that this will make those stem cells, and their progeny, resistant to HIV. The modified cells will then be reintroduced into the patient with the hope that they will create a new, AIDS-resistant immune system.

'While we have a number of drugs that are effective in holding HIV at bay, we have nothing that cures it," says John Zaia, M.D., the Principal Investigator in the trial at City of Hope. "In addition, for many patients, these medications come with significant long-term problems so there is a real need for a therapy that can help eradicate the virus from a patient completely. That is where our work is focused."

CIRM is investing $5.6 million as part of its Strategic Partnership Award to fund the trial. Sangamo is matching that amount.

"There are more than one million Americans living with HIV/AIDS today," says Jonathan Thomas, Ph.D., J.D., Chair of the CIRM governing Board. "This kind of work may be a first step towards finding a cure for them. And while the research is being done here in California the potential impact is clearly a global one. That's why we have been supporting this project for the past five years, through early testing to this point where we can now see if it works in people."

The clinical trial will include people with HIV/AIDS who have had a poor response to standard therapies. This first phase will determine if this approach is safe for these patients, to evaluate what, if any, side effects there may be and to see how the gene-modified stem cells perform when returned to the body. This program extends work by Sangamo, which has safely treated over 70 HIV patients using the same technology to cut out CCR5 in mature T-cells.

CIRM is also funding another trial using an approach similar to this but which employs a different method to disable the CCR5 receptor. Calimmune, an HIV gene medicines company focused on developing cell-based therapies for HIV, began its human clinical trial in July 2013 and has already shown that the first group of patients treated did well enough for the company to start treating a second group more intensively.

For Jeff Sheehy, the CIRM patient advocate Board member for HIV/AIDS and a long-time community activist, this latest approach is important for a number of reasons: "The two CIRM-funded trials are using gene and cell therapy to try and replicate the case of the Berlin Patient, Timothy Brown, who was cured after getting a stem cell transplant from an HIV-resistant donor. This trial is enrolling HIV patients whose immune cells have not returned to normal levels even after success in suppressing the virus with antiretroviral therapy, and even if it doesn't lead to a cure it could still result in a therapy that offers clinical benefit to patients at risk for opportunistic infections."

"This kind of work is too important to just try one method at a time and sit back and wait to see if it is effective," says Thomas. "We have a mission to find treatments for patients in need. By trying several different approaches, taking several shots at goal at the same time if you like, we feel we have a better chance of being successful."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and to act with a sense of urgency commensurate with that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to reality.
